

National Marrow Donor Program®  
**Insert VI – Mucopolysaccharidoses**  
**Annual Follow-up Visit of Recipient**

**Registry Use Only**

Sequence Number:

Date Received:

**Unrelated**

Recipient NMDP ID:  -  -

Recipient Last Name:

Recipient Local ID (optional):

Today's Date:  /  /  TC Code:

Month Day Year

Date of Transplant for which this form is being completed:  /  /

Month Day Year

Visit:  Form 140 —  1 year  2 year

Form 150 —  year

Product type:  Marrow (Form 140/150)  PBSC (Form 540/550)  Cord blood (Form 640/650)

**This form must be accompanied by Form 140, 540, 640 – 6-Month to 2-Year Follow-Up Visit or Form 150, 550, 650 – Yearly Follow-Up Greater Than Two Years Post-Transplant. All information in the box above, including the date, should be identical with the corresponding Form 140/150, 540/550, 640/650. Information should come from an actual examination by the Transplant Center physician, or the physician who is following the recipient post-transplant, or abstraction of the recipient's medical records.**

1. Indicate the enzyme that was evaluated for activity level since the last follow-up. The enzyme reported on this form must correspond to the enzyme found deficient at diagnosis

**Mucopolysaccharidosis**

- 1   $\alpha$ -L-iduronidase (Hurler – MPS I)
- 2  Iduronate sulfatase (Hunter – MPS II)
- 3  Heparan N-sulfatase (Sanfilippo A – MPS IIIA)
- 4   $\alpha$ -N-acetylglucosaminidase (Sanfilippo B – MPS IIIB)
- 5  Acetyl CoA:  $\alpha$ -glucosaminide acetyltransferase (Sanfilippo C – MPS IIIC)
- 6  N-acetylglucosamine 6-sulfatase (Sanfilippo D – MPS IIID)
- 7  Galactose 6-sulfatase (Morquio A – MPS IVA)
- 8   $\beta$ -galactosidase (Morquio B – MPS IVB)
- 9  N-acetyl galactosamine 4-sulfatase (Maroteaux-Lamy – MPS VI)
- 10   $\beta$ -glucuronidase (Sly syndrome – MPS VII)

**Other Storage Diseases**

- 11  Glucocerebrosidase (Gaucher)
- 12  Acid sphingomyelinase (Niemann-Pick)
- 13  Phosphotransferase (Mucopolipidosis II or I-cell)
- 14  Acid lipase (Wolman)
- 15   $\alpha$ -fucosidase (Fucosidosis)
- 16  Neuronal ceroid-lipofuscinosis enzyme – NCL 1 (infantile): PPT-palmitoyl protein thioesterase
- 17  Neuronal ceroid-lipofuscinosis enzyme – NCL 2 (classic late infantile): transpeptidase
- 18   $\alpha$ - or  $\beta$ -mannosidase (Mannosidosis)
- 19  Aspartyl glucosaminidase (Aspartylglucosaminuria)
- 20  Hypoxanthine-guanine phosphoribosyltransferase (Lesch-Nyhan)
- 21  Other storage disease, specify: \_\_\_\_\_

2. Record the enzyme levels:

a. Patient enzyme level:  •  1  nmol/hr/mg protein 2  pmol/hr/mg protein

b. Date of patient test:  /  /

Month Day Year

**Mail to NMDP Registry with Form 140/150, 540/550, 640/650.  
 Retain a copy at the transplant center.**

Recipient NMDP ID:    -    -

Recipient Last Name:

3. Was treatment given for the disease since the last report?

- 1  yes  
2  no  
3  unknown

4. Specify:

- a. Enzyme replacement: 1  yes 2  no 3  unknown  
b. Substrate deprivation/inhibitor: 1  yes 2  no 3  unknown  
c. Gene transfer/gene therapy: 1  yes 2  no 3  unknown

### Clinical Status Post-Transplant

5. Was cerebrospinal fluid (CSF) testing done since the last report?

- 1  yes  
2  no  
3  unknown

6. Report results of most recent tests:

- a. Opening pressure:    cm H<sub>2</sub>O  
1  yes  
2  no  
3  unknown
- b. Total protein:    .   1  mg/dL 2  g/L  
1  yes  
2  no  
3  unknown
- c. Serum albumin:   .  1  mg/dL 2  g/L  
1  yes  
2  no  
3  unknown
- d. Serum globulins:   .  1  mg/dL 2  g/L  
1  yes  
2  no  
3  unknown

7. Date of most recent test:

Month Day Year

8. Was Magnetic Resonance Imaging (MRI) of the brain/spine done since the last report?

- 1  yes  
2  no  
3  unknown

9. Specify location of abnormalities:

*(If possible, attach a copy of the report)*

a. Ventricular (hydrocephalus):

- 1  yes  
2  no  
3  unknown

b. Odontoid hypoplasia:

- 1  yes  
2  no  
3  unknown

c. Other, specify: \_\_\_\_\_

- 1  yes  
2  no  
3  unknown

10. Date of most recent test:

Month Day Year

Recipient NMDP ID:  -  -

Recipient Last Name:

11. Was a Mental Development test done since the last report?

- 1  yes  
2  no  
3  unknown

12. Indicate test instrument; report results of most recent test; report score, not percentile:

- 1  Bayley Scales of Infant Development  
2  Stanford Binet Intelligence Scale 4th ed  
3  Wechsler Preschool and Primary Scale of Intelligence (WPPSI – Revised)  
4  Wechsler Intelligence Scale for Children – III (WISC – III)  
5  Other, specify: \_\_\_\_\_

13. Date of test:

/  /   
Month Day Year

14. Full scale score:

15. Verbal score:

16. Performance score:

17. Were the Vineland Adaptive Behavior Scales done since the last report?

- 1  yes  
2  no  
3  unknown

18. Score results:

a. Communication skills:

b. Daily living skills:

c. Socialization skills:

19. Date of test:

/  /   
Month Day Year

20. Was an eye exam done since the last report?

- 1  yes  
2  no/unknown

21. Visual acuity:

a. Right eye:  /

b. Left eye:  /

22. Was corneal clouding present?

- 1  yes  
2  no  
3  unknown

23. Date of test:

/  /   
Month Day Year

Recipient NMDP ID:    -    -

Recipient Last Name:

24. Was an audiologic evaluation (auditory brain stem or conditioned response) done since the last report?

- 1  yes
- 2  no
- 3  unknown

25. Tympanometry results:

	Normal	Retracted	Flat
a. Right ear:	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>
b. Left ear:	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>

26. Was the hearing loss (HL) in decibels (dB) assessed at the speech threshold for 500 hertz (HZ) since the last report?

- 1  yes
- 2  no
- 3  unknown

27. Speech Threshold results at 500 HZ:

	Normal - Mild	Moderate - Moderately Severe	Severe - Profound
a. Right ear:	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>
b. Left ear:	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>

**See Degree of Hearing Loss chart below for scale ranges.**

28. Was the hearing loss (HL) in decibels (dB) assessed at the speech threshold for 2000 hertz (HZ) since the last report?

- 1  yes
- 2  no
- 3  unknown

29. Speech Threshold results at 2000 HZ:

	Normal - Mild	Moderate - Moderately Severe	Severe - Profound
a. Right ear:	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>
b. Left ear:	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>

**See Degree of Hearing Loss chart below for scale ranges.**

**Degree of Hearing Loss: Pure tones and speech testing**

Normal:	0-20 dB HL	Moderately Severe:	60-70 dB HL
Mild:	21-40 dB HL	Severe:	75-90 dB HL
Moderate:	41-55 dB HL	Profound:	> 90 dB HL

30. Has there been a change in the neurologic status since the last report? (Clinical status, not neuro-psych status.)

- 1  yes
- 2  no
- 3  unknown

31. Status is:

- 1  improved
- 2  worse

If possible, please provide documentation of this change (e.g., physical examination, neurologic examination report).

32. Was pulmonary function testing done since the last report?

- 1  yes
- 2  no
- 3  unknown

33. Oxygen saturation on room air:    %

34. Results of most recent pulmonary function test:  
(If possible, attach a copy of the report)

- 1  normal
- 2  abnormal
- 3  not done

Recipient NMDP ID:    -    -

Recipient Last Name:

35. Was an echocardiogram done since the last report?

- 1  yes →
- 2  no
- 3  unknown

36. Valvular insufficiency:

- a. Tricuspid:
  - 1  none
  - 2  mild or trivial
  - 3  moderate or severe
  - 4  valve replacement
- b. Mitral:
  - 1  none
  - 2  mild or trivial
  - 3  moderate or severe
  - 4  valve replacement
- c. Aortic:
  - 1  none
  - 2  mild or trivial
  - 3  moderate or severe
  - 4  valve replacement
- d. Pulmonary:
  - 1  none
  - 2  mild or trivial
  - 3  moderate or severe
  - 4  valve replacement

37. Date of test:   /   /     
Month Day Year

38. Was the cardiac contractility tested since the last report?

- 1  yes →
- 2  no
- 3  unknown

39. Ejection fraction:   %

40. Shortening fraction:   %

41. Was orthopedic surgery performed since the last report?

- 1  yes →
- 2  no
- 3  unknown

42. Specify site(s):

- 1  Knees
- 2  Hips
- 3  Spine
- 4  Fingers
- 5  Wrist (carpal tunnel syndrome)
- 6  Other, specify: \_\_\_\_\_